EFSPI

European Federation of Statisticians in the Pharmaceutical Industry

Promoting Professional Standards in Europe

Roche

Webinar 2: Estimands – emerging questions now that we are using the framework

5th EFSPI regulatory workshop

13th October 2020

Kaspar Rufibach, on behalf of EFSPI and the organizing committee

We have put you on mute.

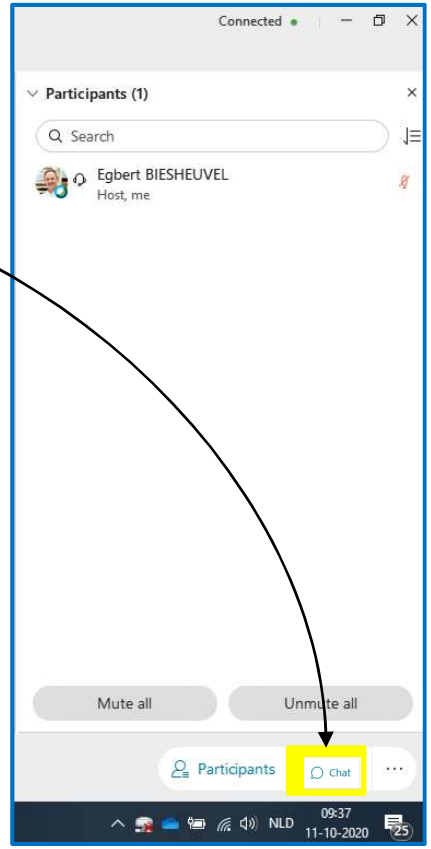
We are waiting for other attendees to join.

We will start soon

Logistics

- We have put all attendees (except for the speakers) on mute

- We will have about 725 participants
- You can send queries through the Chat function:
  - wrt presentation at hand (e.g., clarification), or
  - for panel discussion



- As follow-up all queries will be answered by the presenter if not already addressed during the meeting (Q&A doc)
- Hardly no time for questions between the presentations

- All speakers have agreed to sharing slides and live recording, these will be available at the EFSPI website in due time

5th EFSPI regulatory statistics workshop
13th October 2020

- Organization: EFSPI and organizing committees THANKS!
- Host: Roche (in particular Kaspar Rufibach) THANKS!

Organizing committees

- Local organizing committee

Egbert Biesheuvel (Danone)

Hans Ulrich Burger (Roche)

Christoph Gerlinger (Bayer)

Kaspar Rufibach (Roche)

Emmanuel Zuber (Novartis)

- Scientific committee

Andreas Brandt (BfArM)

Randi Gron (Novo Nordisk)

Maria Gruenewald (Swedish Medical Products Agency)

Cecilia Hedlund (Swedish Medical Products Agency)

Lorenzo Hess (Swissmedic)

Benjamin Hofner (Paul-Ehrlich Institute)

Armin Koch (Medizinische Hochschule Hannover)

Khadija Rantell (MHRA)

Kit Roes (Radboud UMC)

Ina-Christine Rondak (EMA)

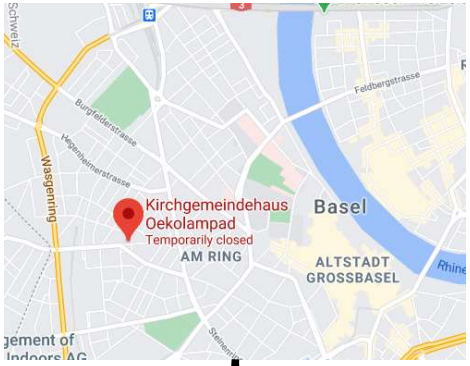
Aldana Rosso (Danish Medicines Agency)

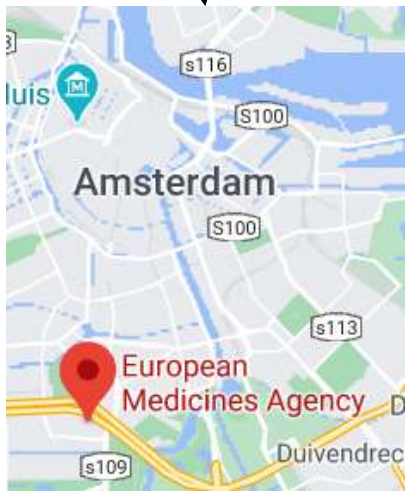
Anja Schiel (Norwegian Medicine Agency)

Steven Teerenstra (Radboud UMC)

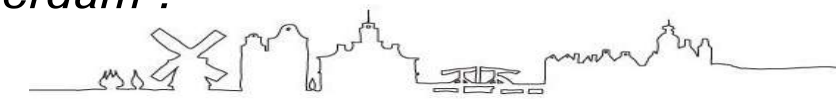
5th EFSPI regulatory statistics workshop

- Jubilee
- After 4 successful years in Basel
- 5th to take place in Amsterdam
- New place of EMA
- However, COVID-19 got in the way



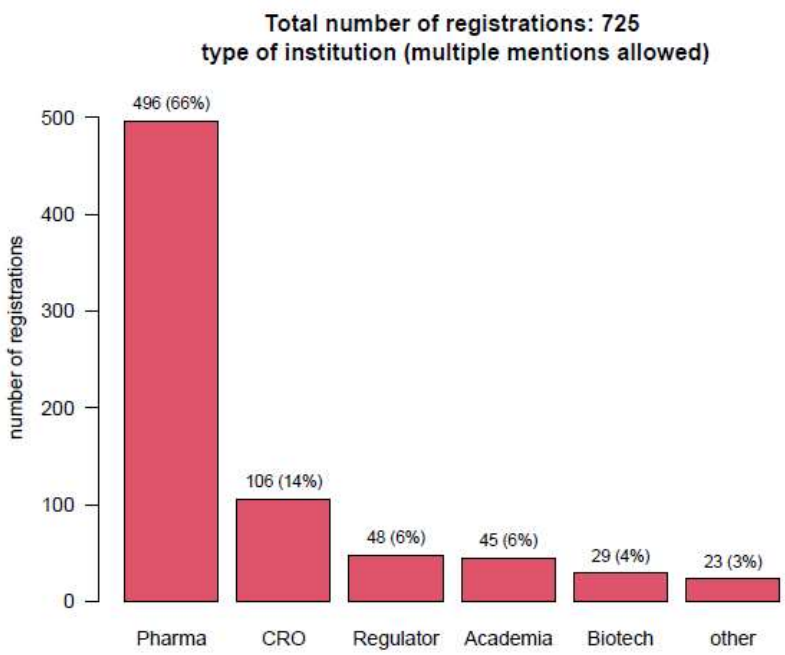


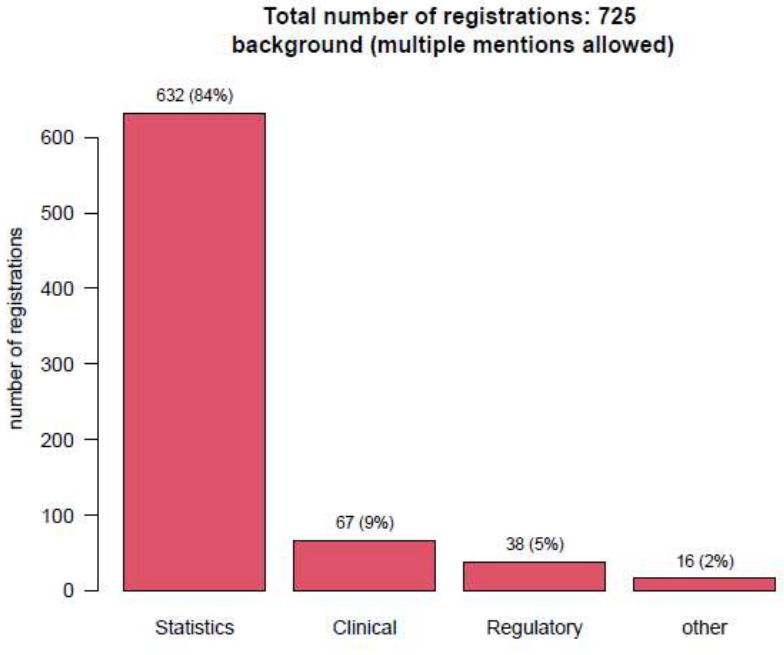
- *Webinar 1: DMCs (yesterday)*
- ***Webinar 2: Estimands (today)***
- *2021 – Amsterdam ?*



Increased number participants

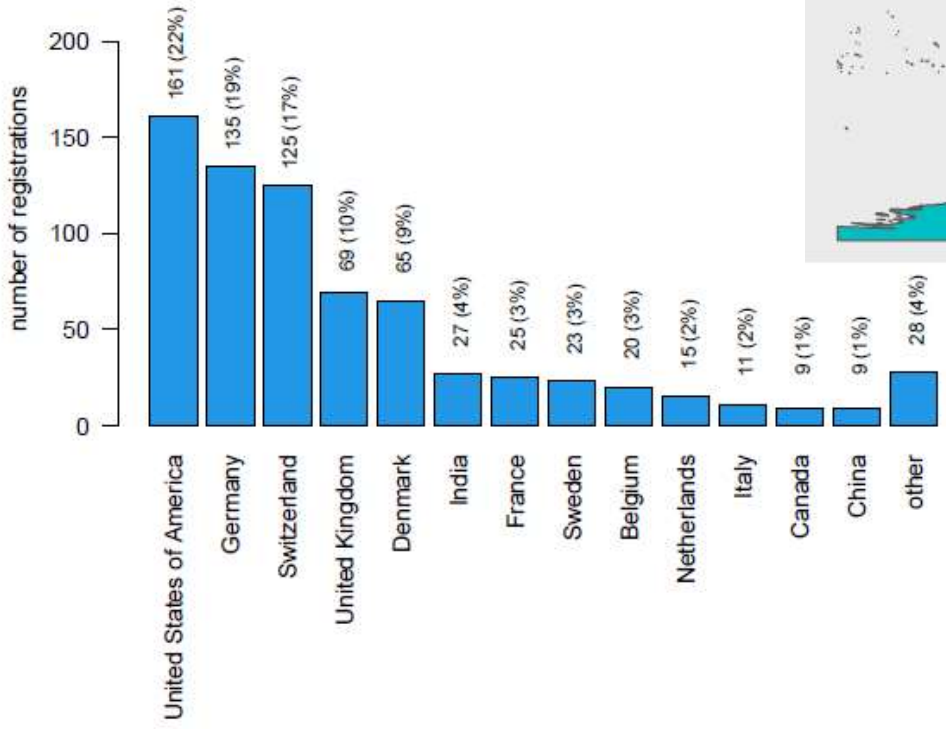
from 200+ live workshop to ≈ 700 people in webinar

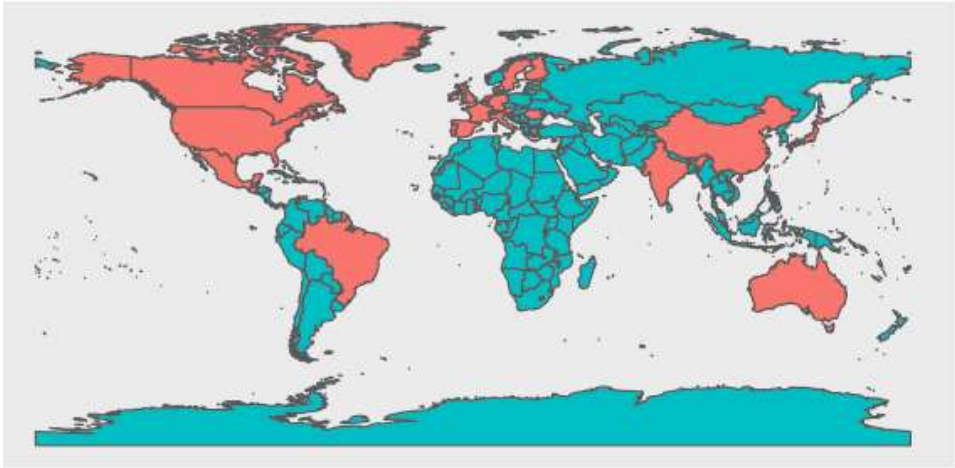




Increased worldwide coverage

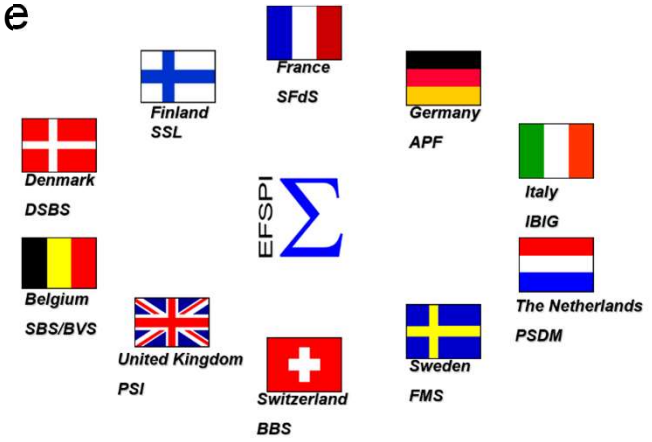
from mainly EU live workshop to “across the globe” webinar





What is EFSPI?

- EFSPI = European Federation of Statisticians in the Pharmaceutical Industry
- Founded in 1992
- A federation of National European Groups
- Now have 10 national groups
- No individual members
- EFSPI is an “umbrella”, non-profit making organisation
- Our national organisations collectively represent 2200 members
- Each organisation has 2 members on the EFSPI Council



- Website: www.efspi.org

EFSPI Activities

- Regulatory Affairs (joint committee with PSI)
  - Organise annual EFSPI Regulatory Statistics workshop
  - Co-ordinates review of regulatory guidance within EU Statistical Community
  - Meet with EU Biostatistics Working Party (BSWG) annually to discuss hot topics
  - Meet with other statistical agencies, e.g. MHRA
  - Hold workshops to discuss draft guidance where appropriate
  - Works with Scientific to identify topics for scientific debate
  - EFSPI is recognized official body in EMA database

Estimands

ICH E9 (R1) Addendum on *Estimands and Sensitivity Analyses in Clinical Trials*, to the guideline on statistical principles for clinical trials

- ICH Final Concept paper, October 2014:
  Choosing Appropriate Estimands and Defining Sensitivity Analyses in Clinical Trials
  - https://database.ich.org/sites/default/files/E9-R1_EWG_Concept_Paper.pdf
- Open for consultation, September 2017
  - https://www.ema.europa.eu/en/documents/scientific-guideline/draft-ich-e9-r1-addendum-estimands-sensitivity-analysis-clinical-trials-guideline-statistical_en.pdf
- ICH final version adopted, November 2019
  - https://database.ich.org/sites/default/files/E9-R1_Step4_Guideline_2019_1203.pdf
- EMA, date coming into effect, July 2020
  - https://www.ema.europa.eu/en/documents/scientific-guideline/ich-e9-r1-addendum-estimands-sensitivity-analysis-clinical-trials-guideline-statistical-principles_en.pdf
- EFSPI regulatory workshops: sessions on *Estimands* in all previous events

Estimands – emerging questions now that we are using the framework

Agenda – Webinar 2

13th October 2020 - Webinar 2: Estimands – emerging questions now that we are using the framework

Registration link:
https://docs.google.com/forms/d/e/1FAIpQLSdIqa1_T9IqMboJwOO3vAHrhp9TbNKKLX4AO2beFgwVfBtdGA/viewform

14:00 – 14:10 **Egbert Biesheuvel (EFSPI council member and "local" organizing committee)**
Welcome and scene setting

14:10 – 14:30 **Finbarr Leacy (Health Products Regulatory Authority, Ireland)**
Regulatory update: learnings, planned guideline updates, and recommendations & asks for industry

14:30 – 14:50 **Vivian Lanius (Bayer), Armin Schüler (Merck KGaA), David Wright (AstraZeneca)**
Feedback from EFSPI / EFPIA estimand implementation working group

14:50 – 15:00 **Break**

15:00 – 15:45 **Impact of COVID-19 on clinical trials and estimands: examples and general considerations**
Yongming Gu (Eli Lilly): Using a mix of strategies in handling intercurrent events and missing values for studies impacted by the COVID-19 pandemic

Guenther Mueller-Velten, Yi Wang, Melanie Wright (Novartis): Impact of COVID-19 and risk mitigation in a global cardiovascular outcomes trial

15:45 – 16:30 **Panel discussion**
All speakers

16:30 – 16:35 **Kaspar Rufibach ("local" organizing committee)**
Closure

We look forward to your participation!

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Kaspar Rufibach, on behalf of EFSPI and the organizing committee

BREAK!

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Reflections to start Panel discussion:

- **Finbarr Leacy**: shared his experiences by today as a regulator, impact of COVID-19, and future development including engaging our clinical colleagues.

- **David Wright and Vivian Lanius**: showed us the purpose, ongoing activities and future work of the Estimand Implementation Working Group, including the 'patchwork' at the moment.

  "The estimand framework provides a comprehensive approach to articulate this impact analysis." make.

- **Yongming Gu**: explained to us that a mix of strategies to deal with ICEs might be more appropriate than the more common approach to use only ONE strategy for all ICEs.

- **Guenther Mueller-Velten**: demonstrated the impact of COVID-19 on a concrete cardiovascular trial, and the considerations you have to make.

As follow-up all queries will be answered by the presenter

Back Up